PHARMAC
TE PĀTAKA WHAIORANGA

Submission form for consultation on paediatric cancer treatments in New Zealand

In addition to standard background information, we have provided focus questions to help shape your feedback. You don't need to respond to all the questions – only the ones you want to. The final question is open for you to add anything that hasn't been covered by the earlier questions.

Background information

Your name
Lucy Elwood, Chief Executive

The name of the group or organisation you represent

Cancer Society of New Zealand

Are you one or more of the following?
(Tick as many boxes as appropriate)
☐ Someone who has been treated for cancer as a child
☐ Family or whānau of someone who has been treated for cancer as a child
☐ A professional working with children who have cancer
☐ A paediatric cancer researcher
☐ Other

How old are you?
☐ 14 years or younger
☐ 15 to 30 years
☐ 31 to 50 years
☐ 51 years or older

Which of these ethnic groups do you strongly identify with?
(Tick as many boxes as appropriate)
☐ Māori
☐ Pacific peoples
☐ Asian
☐ Middle Eastern / Latin American / African
☐ European and other

Contacting you

If you choose, you can provide an email address
Lucy@cancer.org.nz

If you've provided an email address, we will be in touch to confirm we have received your submission and tell you when a summary of submissions has been published online.

Let us know if you we can contact you:
☐ to seek further clarification about something in your submission
☐ about information on further steps to be taken following this stage of the review
☐ about general news and updates from Pharmac

Your feedback may be shared

We will only use your information to inform our review of rule 8.1. Feedback we receive is, however, subject to the Official Information Act 1982 (OIA). Please be aware that we may need to share your feedback, including your identity, in response to an OIA request.

If you want any part of your feedback treated as confidential, you need to tell us which information and why. For example, would you like us to withhold your identity or a particular part of your submission because it is personal, confidential, commercially sensitive, or proprietary? If your request meets the Official Information Act criteria we won't be required to disclose it.

Pharmac's privacy statement

Is there anything in your submission you would like us to consider withholding and why?
Click or tap here to enter text.

How well do we understand child cancer and the system of care?

Is our understanding of the overall health outcomes being achieved for people with paediatric cancers correct?
If not, please provide any further information or context

As a cancer non-governmental organisation (NGO) we primarily serve adults with cancer and their whānau. Our services to children are generally focused on impacts on children following an adult cancer diagnosis. We do work alongside Canteen to support whānau where an adolescent or young adult (AYA) has cancer. We understand that Child Cancer Foundation and Canteen are providing detailed comment, as lead providers to children and AYA with cancer, and encourage you to engage actively with them on the overall health outcomes being achieved for children with cancer.

In what other clinical contexts is participation in clinical trials the 'standard of care'?
Clinical trials are an important part of the health system. In the field of cancer treatment, it is increasingly common to subset malignancies according to specific genetic changes, and to personalise therapies accordingly. These personalised therapies are assessed in 'platform trials', in which a number of new medicines are assessed, each in the small number of patients who are most likely to benefit from them. Niche medicines, such as kinase inhibitors or immunotherapies, are often manufactured by smaller pharmaceutical

companies without representation in NZ. These 'platform trials' are often run by international co-operative groups, which are non-profit organisations.

In light of this practice, it is critical to have a rapid access scheme so that adults with life-threatening cancer are not disadvantaged by a slow and inefficient approval process. Access to clinical trials is not fairly distributed across population groups, most clinical trials are located in major centres so are largely unattainable for people living rurally or in areas of high deprivation, as well as for Māori.

The work of the National Child Cancer Network (NCCN) can be viewed as a model for other parts of the health system, particularly in the area of adult cancer care. This high performing and well-established hub and spoke approach has set a high bar for other subspecialities in cancer care.

To what extent is access to paediatric cancer clinical trials dependent on access to medicines through rule 8 1b?
Undoubtedly application of rule 8.1b has helped facilitate timely, and effective clinical trials of new paediatric cancer medicines. Any move to end rule 8.1b could fundamentally change paediatric cancer care in New Zealand. We would be deeply saddened by this, as it is an area where New Zealand's cancer survival rates are comparable with those from other jurisdictions. In many other cancers, medicine funding for life saving cancer treatments in New Zealand is significantly below other jurisdictions. In their report 'Understanding the gap: an analysis of the availability of cancer medicines in Aotearoa', Te Aho o Te Kahu identified 18 medicines publicly available in Australia but not in New Zealand, of these three were curative in nature. Paediatric cancer treatment outcomes are an area where the health system can demonstrate more equitable outcomes for Māori and Pacific children. This should be seen as a great example of a working health system and continued, rather than considering a reduction in access to these medicines via rule 8.1b.

How sensitive is this system of care to changes to rule 8 1b?
See above.

How effective is rule 8.1 in terms of achieving the best health outcomes?

To what extent are good health outcomes for children with cancer in New Zealand dependent on making paediatric cancer treatments available through rule 8 1b?
To a large extent. Speaking very generally Pharmac's processes rely on evidence of clinical benefit, which require clinical trials to be completed to evidence that benefit. A change to rule 8.1b would limit access to such trials for children with cancer. For any treatment proven effective in a clinical trial, New Zealand children would then not be included during those clinical trials and be left waiting for good evidence to fund the medicine publicly. Participation of the NCCN in the clinical trial also enables treatment pathways using new medicines to be trialled and implemented to achieve a more consistent approach. Pharmac has recently had substantial discussions with Te Whatu Ora about how to prepare the health system to provide immunotherapy for people with lung cancer – in comparison to the work of the NCCN, these discussions occur organically through senior medical officers' informal networks and their involvement in the trial.

Is timely access to paediatric cancer treatments more important than timely access to other medicines or for other populations? If so, why?
Timely access to cancer medicines is important for all populations. However, the QALY calculations for paediatric patients, adolescents and young adults will inevitably support funding of even very expensive new treatments. The level of analysis that is therefore

needed to decide funding decision can be less robust as a consequence, because the QALYs for paediatric treatments will be high and compelling for any treatment that is either (a) curative; or (b) which halts progression of a degenerative or life-threatening condition.

Does the current policy support efficient and sustainable use of available resources?

Is our understanding of how rule 8 1 operates in practice correct? What else should we know?

We do not have any concerns about the efficiency and/or effectiveness of rule 8.1b. In general terms, our tamariki are quickly transferred to the appropriate centre for cancer treatment and children across New Zealand achieve broadly comparable outcomes. NGOs like Child Cancer, Canteen and Ronald McDonald House Charities (RMHC) provide important wrap-around support. Rule 8.1b contributes to demand for their services. For example, if rule 8.1b was revoked and new paediatric cancer treatments followed a similar path for funding decisions as for new adult cancer treatments, we would have substantial concerns. Without this exception, unfunded treatments cannot be provided in public hospitals. We are not currently aware of any private paediatric cancer treatment providers, because the public system is performing well. It would be of great concern if a change to the funding regime created an incentive for private paediatric cancer treatment providers to be established – this would create a two-tier system; drive inequities; and also have considerable adverse flow on consequences to cancer support providers (e.g. RMHC houses would not likely be located close to private treatment centres or as well placed to provide support). It has been our experience in supporting adults with cancer accessing treatment privately, that they are unable to access other publicly funded services (e.g. CPSSS, allied health, cancer nurse specialist for their cancer type).

How much increase in the use of rule 8 1b do you think will happen as a result of the growing range of new paediatric cancer treatments?

We do not believe that there is a material concern. While costs have been increasing, they should reach a natural limit which is below a point of public policy concern. This is because of the relatively small number (approximately 150 per annum) of children diagnosed with cancer each year and the conditions for each cancer treatment.

Do you see the costs of paediatric cancer treatments accessed through rule 8 1b increasing significantly in the foreseeable future?

We do see the potential for costs to increase, including as CAR-T cell treatments move from a limited clinical trial to part of a recognised cancer pathway for some child cancers. (We have attached the National Services Framework CAR-T cell therapy application dated February 2023.) However, as noted above the QALYs for curative paediatric medicines will support this investment.

How could we assess what value paediatric cancer treatments provide against other medicines that could be funded with the same money?

A QALY comparison could be done on a periodic basis, if considered necessary to give public confidence. However, we have not seen any evidence of the public being concerned about investments in paediatric cancer.

Does the current policy support equity?

What should Pharmac take into account when considering equity issues with respect to rule 8 1b of the Pharmaceutical Schedule?

Yes, the current policy supports equity. The gap between cancer outcomes for different population groups by ethnicity in child cancer is small.

When AYA with cancer are not treated by a paediatric cancer centre (so can't access clinical trials under rule 8.1b), we understand there is more concern about equitable access to new cancer treatment and treatment outcomes. Canteen and Te Aho o Te Kahu may be able to provide input.

Do you consider rule 8 1b to be inequitable from the perspective of other children or those with rare disorders? Why?

We're concerned that the current funding process for other children, especially children with rare disorders is too slow in New Zealand compared to other jurisdictions. We would support extending rule 8.1b to include other paediatric conditions which will inherently result in large QALYs (e.g. where the condition is quickly degenerative or life threatening). We know New Zealanders want better and quicker funding of medicines. Spinraza took too long to be funded in New Zealand, and affected children missed out as a result of those delays.

As the NCCN has demonstrated with regard to rule 8.1b, a well-functioning clinical network is key. There will be some very rare conditions where a condition-specific network is not possible because of the very small number of patients in New Zealand. A paediatric rare disorder network could be established to advise and source guidance from international colleagues on clinical efficacy and treatment paths. An added benefit could potentially be consistent clinical pathways for less common conditions.

Another alternative is to establish a very rapid access scheme for paediatric cancer medicines. A rapid access scheme would deliver the following benefits:

- access to promising medicines sooner than standard assessment processes
- Provide clarity, certainty and transparency of route which makes it easier for stakeholders
- May stimulate more clinical trials in Aoteoroa New Zealand.

We have attached an NZIER paper on rapid access schemes (*Briefing notes to Medicines NZ. Rapid Access to new medicines in NZ*. Wellington. NZ Institiute of Economic Research/NZIER) and would also encourage Pharmac to consider the success of the relatively new innovation fund in the UK launched in 2019.

We strongly support a rapid access scheme also being available for decisions about other cancer medicines (e.g., for new adult cancer treatments) and potentially the introduction of a paediatric rapid access scheme for medicines for rare paediatric conditions could be a useful initial pilot of a new scheme; with later expansion into other funding decisions for new medicines.

To what extent do the current policy settings, including rule 8 1b, contribute to the health outcomes achieved for tamariki Māori and Pacific children with cancer?

Rule 8.1b does contribute to improved outcomes. New Zealand compares well with other jurisdictions in regard to paediatric cancer outcomes (compared to other groups) and the performance across all patient groups and settings (e.g. children living rurally) are better than other cancer types.

Do you consider rule 8 1b to be inequitable from the perspective of adolescent and young adults with cancer? Why?
Yes. Where a young adult or adolescent with cancer is being treated by adult cancer services, they are subject to the same slow process as funding for new adult medicines. The QALYs for this group will also be significant, and improving access for AYA should be a priority area. This could be done through a rapid access scheme, as discussed above.

How might we address equity and fairness concerns related to paediatric cancer medicines through rule 8 1b and access to medicines for other groups?
See above re AYA, and comments for a rapid access scheme for new cancer medicines for adults.

Other information or thoughts?

Is there anything else we need to know to inform the review? If so, please add your information or thoughts here
Child cancer is a very emotive area. If Pharmac looked to change its policies, there would be considerable adverse reaction. Any actual change that resulted in delayed access to medicines for a child with cancer would give rise to substantial negative media and risk to reputation.